A Novel Treatment for Fragile X Synaptic and Cognitive Deficits

Published date: Aug. 14, 2019

Technology description

Fragile X syndrome (FX) is the most common form of inherited intellectual disability and occurs in roughly 1:3,600 males and 1:8,000 females. Physical features associated with FX include increased dendritic spine density, long faces, large testicles, and connective tissue problems such as flat feet, double-jointed fingers and hyper-flexible joints. Behavioral characteristics can include ADD, autism and social anxiety. While there is nosingle treatment for FX, there are palliative treatments that are aimed at improving the lives of affected individuals as opposed to treating the underlying condition. There are various clinics testing new medications, but no FDA-approved drug is currently availablespecifically for treating FX.

Breakthrough

Based on a finding that mice with FX exhibit excessive vasculature in primary visual cortex, the Galvez Lab at the University of Illinois at Urbana-Champaign has significantlyreduced both physical and cognitive abnormalities in affected mice by controlling the expression of vascular endothelial growth factor-A (VEGF-A), the dominant regulator of vascular growth. This treatment in mice demonstrates a reduction in characteristics

common in FX affected individuals and has direct relevance to the underlying mechanisms of these abnormalities as well as a potential treatment of the syndrome.

Design

The Galvez Lab has found that VEGF is overexpressed in FX mice. To explore the role of VEGF-A in mediating other FX abnormalities, VEGF-A was blocked in adult FX mice using

Bevacizumab, a drug used to block new blood vessel formation. After 10 days, FX mice treated with the drug exhibited significant reductions in testicle weight and a decrease in synapse density, which strongly suggests elevated VEGF-A expression is a majorcontributor for some FX abnormalities. Due to the effect of Bevacizumab on FX synapse abnormalities, the Galvez Lab also examined effects on cognitive functions via a novel vs.familiar object preferential test. The findings demonstrate that VEGF-A contributes not only to physical abnormalities of FX but to associated cognitive abnormalities as well. Further studies are being done to better understand FX and to treat the condition itself.

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